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Genetix Pharmaceuticals Announces Global Clinical Plans to Treat Adrenoleukodystrophy (ALD)

Gene Therapy Technology Licensed from National Institute of Health and Medical Research (INSERM) Featured in November Issue of Science

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genetix Pharmaceuticals, a leader in gene therapy of somatic stem cells, announced today the company's clinical program to treat Adrenoleukodystrophy (ALD). The company is developing a gene therapy product in collaboration with Professor Patrick Aubourg and Doctor Nathalie Cartier of the National Institute of Health and Medical Research (INSERM). Genetix is planning to file an IND to expand the development in the US, as well as to expand the ongoing trial in France. The current development timeline should make the product commercially available to patients by 2014. Patrick Aubourg and Nathalie Cartier will become scientific advisors to Genetix's ALD clinical programs.

ALD is a devastating disease that afflicts 1/17,000 males with onset of severe cognitive and neurologic deficits between the ages of 5 and 12, or in adulthood. It progresses to death within 3-10 years of diagnosis. Many people became familiar with ALD from the 1992 movie *Lorenzo's Oil*, which featured a young boy affected with this disease.

ALD is currently treated with allogeneic bone marrow transplantation when there is an appropriate donor. Even so, the graft sometimes does not take and there is Graft Versus Host Disease leading to death in 15% of children and at least 30% of adults post transplant. For those patients who survive the transplant procedure, the disease is stabilized and no longer progresses. In 50% of the patients who survive, Graft Versus Host Disease or delayed hematological reconstitution delay the time at which disease is stabilized, allowing the disease to have progressed further before stabilization.

Patrick Aubourg, M.D. of Inserm-University Paris Descartes commented, "We are delighted to develop this ground breaking gene therapy product together with Genetix, the leading gene therapy company. Our future close scientific collaboration is off to a good start. Production of large GMP batches is underway, which will allow us to rapidly treat the rest of the patients in this clinical trial. We look forward to working with Genetix to expand the trials and acquire the pivotal data and regulatory approvals needed to bring this product to market."

Gene therapy places a healthy gene into the patients' own bone marrow stem cells. In the first two patients described in the November issue of *Science*, the disease has been stabilized. To view the *Science* article, click on the following link: <http://www.sciencemag.org/cgi/content/abstract/326/5954/818>

Genetix's gene therapy approach has the following advantages over allogeneic bone marrow transplantation:

- Applicable to all patients and no limitation to have a matched donor;
- No risk of death from Graft Versus Host Disease by using patients' own cells;
- More rapid engraftment/reconstitution of marrow and immune function post transplant due to perfect match between patients' stem cells and marrow milieu.

To date, in both patients treated, the therapy has been safe and efficacious. As with all drugs in development, the ongoing

clinical trials are designed to statistically prove safety and efficacy for regulatory approval.

Alfred Slanetz, Chief Executive Officer of Genetix, commented, "We congratulate our colleagues and collaborators at INSERM on their publication and accomplishments. Today two of two treated children with ALD, who otherwise would be severely cognitively impaired and on a road to death, are able to live healthy and normal lives because of the ALD lentiviral gene therapy. Developing safe and effective treatments for incurable diseases is what our industry is all about. Genetix is now committed to developing this product in the most scientific and ethical way, and to make this product commercially available to all patients afflicted with this devastating disease."

About Genetix Pharmaceuticals

Genetix is a biopharmaceutical company with operations in Cambridge, MA and Paris, France. Its products are curative and treat the cause of genetically based diseases by placing a healthy gene into the patient's extracted bone marrow or other somatic stem cells, and transplanting these corrected cells back into the patient. The Genetix clinical pipeline includes: Lenti-D for Adrenoleukodystrophy (ALD) now in development, and Lentiglobin for Thalassemia/Sickle Cell Disease also in clinical development with projected launch in 2014 and 2015, respectively. The company is a leader in the gene therapy of somatic stem cells, GMP production and product development. Its proprietary platform technology is applicable to the development of curative therapies for numerous important diseases. The company's unique technology, production capability and international team enable global reach with modest infrastructure. As the leading global gene therapy company, Genetix is committed to developing curative therapies for the world's patients.

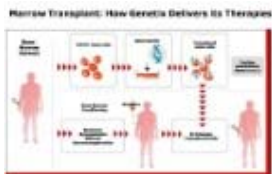
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Graphic

Marrow Transplant: How Genetix Delivers its Therapies (Graphic: Business Wire)



